Clinical Guideline

DUCHENNE'S MUSCULAR DYSTROPHY: MONITORING OF STEROID THERAPY

SETTING Paediatric Neurology, UHBristol

FOR STAFF Paediatric Neurology and Paediatric Endocrinology

PATIENTS Boys with Duchenne's Muscular Dystrophy *(complete and file in patient notes)*

Patient Name and DOB:

Background: Since the 1970s, steroids have been used to slow progression of muscular decline in DMD. As addressed by Cochrane[1] and other reviews[2,3], treatment is effective but uncertainty continues on best practice optimal dose and duration. Steroid therapy has benefits and risks.

Aim: this guideline provides guidance on minimising potential adverse effects of prolonged steroid therapy, covering preventative approaches, investigations and referral pathways.

Recommended actions within Duchenne's clinic:		**Action taken** (√ or NA)
Use minimal effective steroid dose	Appropriate duration of steroid therapy remains controversial [2,3]. Consideration of when to cease steroids needs case by case consideration and discussion. The risk of side effects is linked with cumulative steroid dose[2,3].	
Weight bearing exercise	Encourage levels of activity appropriate for the individual boy's motor functional level. Studies show that even use of a standing frame 30 minutes three times a week has beneficial effects on bone mineralisation and development.	
Ensure child is on Vitamin D supplements	Boys with Duchenne's treated with steroids should be routinely prescribed ongoing Vitamin D supplements. This is because the Chief Medical Officer (CMO) recommends that daily Vitamin D supplements be provided to those on long term steroids e.g. prednisolone (expected duration > 6 months). Generally this should be 400 units daily. Adolescents often require 800 units daily. Refer to Guideline on Vitamin D Deficiency in Children for further details and options, as that document also outlines supplementation.	
Consider blood test to assess Vitamin D level	Use clinical judgement. Remember the above mentioned Vitamin D supplements are indicated even without a blood test. A blood test to assess adequacy can be useful to determine if higher doses needed – again see Guideline on Vitamin D Deficiency in Children for further details.	
Lateral spine Xray	If a boy complains of back pain, undertake a Lateral spine Xray to look for vertebral collapse fractures.	

Monitor Growth	Regularly plot height and weight on growth chart	

Referrals to Endocrinology for additional input (after addressing the items on previous page)			
Referral for What?	**When?**	**Details**	**Action taken** (√ or NA)
Dual-energy X-ray Absorptiometry (DEXA)	Please implement the above items first of all. • Useful to obtain baseline at commencement of steroids and after correcting any Vitamin D deficiency (<25 nmol/L) • Thereafter,1-2 yearly if ambulant; non-ambulant 6 monthly • Lateral spine X ray if Z score <2 SD	Request Paediatric DEXA on Medway Service Orders. DEXA scan will be performed on DEXA machine in Dept Rheumatology, Level 6, BRI. The room does have a hoist. Please provide details on whether can self-transfer or will need a hoist on the Medway Service Order, so that DEXA technicians can allocate appropriate timeslot for the scan. Please also note if they have had spinal rodding surgery, as this will influence which views are obtainable on DEXA scanning.	
Tetracosactide (Synacthen) Test	Recommended: • after long duration of steroids • especially if considering reduction or cessation of steroids	Please write to Dr Burren requesting Synacthen test, including details of current steroid dose, approximate duration and any plans for reduction or cessation. Paediatric Endocrinology team will then arrange CIU admission and coordinate the necessary prednisolone to hydrocortisone conversion prior to test.	
Clinical opinion from paediatric endocrinology	Clinical referral recommended if either: • fractures have occurred • or concerns regarding growth and puberty	Please write to Dr Burren outlining clinical details. Patient will generally then be seen in endocrine clinic. Remember, routine DEXAs and Synacthen test's can be requested separately, as above.	

References:

1. Osteoporosis in Duchenne Muscular Dystrophy. Quinlivan et al. Neuromuscular Disorders 2005;15:72-79.
2. Glucocorticoid corticosteroids for Duchenne muscular dystrophy (Review). Manzur et al. The Cochrane Library 2009. DOI: 10.1002/14651858.CD003725.pub3

3. Diagnosis and management of Duchenne muscular dystrophy, part 2: implementation of multidisciplinary care. Bushby et al The Lancet Neurology 2010;9:177-189.

RELATED DOCUMENTS Vitamin D Deficiency in Children
DMS address ie http://nww.avon.nhs.uk/dms/download.aspx?did=14579

QUERIES Contact Dr Christine Burren Tel 0117 342 0203 Fax 0117 342 0187